

(FILE 'HOME' ENTERED AT 13:32:53 ON 07 MAY 2003)

FILE 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, AQUASCI,  
BIOBUSINESS, BIOCOMMERCE, BIOSIS, BIOTECHDS, BIOTECHNO, CABA, CANCERLIT,  
CAPLUS, CEABA-VTB, CEN, CIN, CONFSCI, CROPB, CROPU, DGENE, DRUGB,  
DRUGLAUNCH, DRUGMONOG2, DRUGNL, DRUGU, DRUGUPDATES, ...' ENTERED AT  
13:33:08 ON 07 MAY 2003

L1           251 S HEMATOPOIETIC (1A) SPECIFIC? (3A) EXPRESSION  
L2           109 DUP REM L1 (142 DUPLICATES REMOVED)  
L3           3 S HEMATOPOIETIC SPECIFIC PROMOTER  
L4           5 S L2 AND TISSUE SPECIFIC PROMOTER

L6 ANSWER 59 OF 172 SCISEARCH COPYRIGHT 2003 THOMSON ISI  
AN 94:609210 SCISEARCH  
GA The Genuine Article (R) Number: PH589  
TI PRESENCE OR ABSENCE OF FACTOR-IX IN NORMAL  
PLATELETS  
AU FABRIS F (Reprint); BETTERLE C; GIROLAMI A  
CS UNIV PADUA, SCH MED, INST MED SEMEiot, CHAIR INTERNAL MED 4, VIA OSPEDALE  
105, I-35100 PADUA, ITALY (Reprint)  
CYA ITALY  
SO BLOOD COAGULATION & FIBRINOLYSIS, (AUG 1994) Vol. 5, No. 4, pp. 659.  
ISSN: 0957-5235.  
DT Letter; Journal  
FS LIFE  
LA ENGLISH  
REC Reference Count: 5

L6 ANSWER 60 OF 172 BIOSIS COPYRIGHT 2003 BIOLOGICAL ABSTRACTS INC.  
DUPLICATE 17  
AN 1994:527516 BIOSIS  
DN PREV199497540516  
TI Platelets contain releasable coagulation factor  
IX antigen: Response.  
AU Hoffman, M. (1), Römp, K.; Monroe, D.  
CS (1) Dep. Pathol. Med., Duke Univ. Med. Cent. Univ. North Carolina, North  
CA USA  
SO Blood Coagulation & Fibrinolysis, (1994) Vol. 5, No. 4, pp. 657-658.  
ISSN: 0957-5235.  
DT Article; Letter  
LA English

L6 ANSWER 93 OF 172 SCISEARCH COPYRIGHT 2003 THOMSON ISI  
AN 87:272961 SCISEARCH  
GA The Genuine Article (R) Number: H1787  
TI IDENTIFICATION OF BINDING-SITES FOR FACTOR-IX AND  
FACTOR-IXA ON HUMAN-PLATELETS  
AU AHMAD S S (Reprint); RAWALA R; WALSH P N  
CS TEMPLE UNIV, PHILADELPHIA, PA, 19140  
CYA USA  
SO FEDERATION PROCEEDINGS, (1987) Vol. 46, No. 6, pp. 2244.  
DT Conference; Journal  
FS LIFE  
LA ENGLISH  
REC No References

L6 ANSWER 96 OF 172 BIOSIS COPYRIGHT 2003 BIOLOGICAL ABSTRACTS INC.  
AN 1988:41003 BIOSIS  
DN BR34:18023  
TI INTERACTIONS OF HUMAN PLATELETS WITH COAGULATION FACTORS  
IX AND IXA.  
AU AHMAD S S; RAWALA R; WALSH P N  
CS THROMBOSIS RES. CENT., DEP. MED., TEMPLE UNIV. SCH. MED., PHILADELPHIA,  
PA, USA.  
SO XITH INTERNATIONAL CONGRESS ON THROMBOSIS AND HAEMOSTASIS, BRUSSELS,  
BELGIUM, JULY 6-10, 1987. THROMB HAEMOSTASIS. (1987) 58 (1), 351.  
CODEN: THHADQ. ISSN: 0340-6245.  
DT Conference  
FS BR; OLD  
LA English

L6 ANSWER 99 OF 172 SCISEARCH COPYRIGHT 2003 THOMSON ISI  
AN 87:491215 SCISEARCH  
GA The Genuine Article (R) Number: J6998  
TI FACTOR-VIII MEDIATES BINDING OF FACTOR-IX TO

L4 ANSWER 8 OF 8 WPIDS (C) 2003 THOMSON DERWENT  
AN 2003-157028 [15] WPIDS  
DNN N2003-123898 DNC C2003-040916  
TI New nucleic acid comprising all or part of a gene encoding a procoagulant factor (e.g. **Factor VIII**) operably linked to a megakaryocyte/platelet specific regulatory region, useful in **gene therapy**, especially for treating hemophilia A.  
DC B04 D16 P14  
IN CONWAY, E M; SCHUH, A C  
PA (CONW-I) CONWAY E M; (SCHU-I) SCHUH A C  
CYC 100  
PI WO 2002102850 A2 20021227 (200315)\* EN 34p

09/606927

RW: AT BE CH CY DE DK EA ES FI FR GB GH GM GR IE IT KE LS LU MC MW MZ  
NL OA PT SD SE SL SZ TR TZ UG ZM ZW  
W: AE AG AL AM AT AU AZ BA BB BG BR BY BZ CA CH CN CO CR CU CZ DE DK  
DM DZ EC EE ES FI GB GD GE GH GM HR HU ID IL IN IS JP KE KG KP KR  
KZ LC LK LR LS LT LU LV MA MD MG MK MN MW MX MZ NO NZ OM PH PL PT  
RO RU SD SE SG SI SK SL TJ TM TN TR TT TZ UA UG US UZ VN YU ZA ZM  
ZW

ADT WO 2002102850 A2 WO 2002-CA903 20020617

PRAI US 2001-298121P 20010615

AB WO2002102850 A UPAB: 20030303

NOVELTY - A nucleic acid sequence (N1), which comprises all or part of a gene sequence encoding a procoagulant factor operably linked to a megakaryocyte/platelet specific regulatory region, is new.

DETAILED DESCRIPTION - INDEPENDENT CLAIMS are also included for the following:

- (1) a B-domain deleted form of factor VIII, where residues 761-1630 of human Factor VIII have been deleted;
- (2) a vector for expression of N1;
- (3) a genetically modified cell expressing N1;
- (4) a transgenic animal expressing N1;
- (5) a method of treating hemophilia A comprising:
  - (a) providing a nucleic acid construct comprising the sequence encoding a procoagulant factor operably linked to a tissue-specific promoter;
  - (b) introducing the nucleic acid construct into bone marrow cells to obtain genetically modified cells; and
  - (c) implanting the genetically modified cells into a patient; and
- (6) a method of **gene therapy** by:
  - (a) administering to a patient a viral vector comprising the nucleic acid sequence encoding a Factor VIII gene product, where the expression of the Factor VIII gene product is regulated by a megakaryocyte specific promoter; or
  - (b) (for ex vivo **gene therapy**) administering genetically modified cells expressing a desired gene product, where a megakaryocyte specific promoter is used to regulate the expression of gene product.

ACTIVITY - Hemostatic; Coagulant.

No biological data given.

MECHANISM OF ACTION - **Gene Therapy**.

No biological data given.

USE - The nucleic acid is useful in **gene therapy**, particularly for treating hemophilia A. The vector or the genetically modified cell is also useful in **gene therapy** for treating hemophilia A.

Dwg.0/7

STIMULATED PLATELETS  
AU MUNTEAN W (Reprint); LESCHNIK B  
CS GRAZ UNIV, DEPT PEDIAT, A-8010 GRAZ, AUSTRIA  
CYA AUSTRIA  
SO THROMBOSIS AND HAEMOSTASIS, (1987) Vol. 58, No. 1, pp. 351.  
DT Conference; Journal  
FS LIFE  
LA ENGLISH  
REC No References